UCLA Launches CIRM-Funded Cancer Clinical Trial

Posted: September 11, 2017

Therapy uses engineered blood stem cells and mature T cells to kill hard-to-treat cancers

September 11, 2017 Oakland, CA When a cancer metastasizes, or spreads, to other parts of the body and becomes resistant to available treatments, patients and their doctors have few, if any, alternatives to stop the cancer's deadly growth. Scientists at the Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research at UCLA announced today that they have launched a phase 1 clinical trial testing a novel therapy targeting these hard-to-treat cancers.

The clinical trial is funded by a $20 million grant from the California Institute for Regenerative Medicine (CIRM) and is supported by the CIRM-funded UCLA-UCI Alpha Stem Cell Clinic, part of a network of top California medical centers that specialize in delivering stem cell clinical trials to patients. In August 2017, the Food and Drug Administration (FDA) authorized the UCLA team to begin enrolling patients in this trial.

The therapy takes advantage of the fact that ten to 20 percent of all cancers, such as skin cancer, carry a protein called NY-ESO-1 on their cell surface. Because NY-ESO-1 is rarely found on normal cells, it provides a means to specifically seek out and destroy the cancer cells. In this trial, the scientists will collect blood-forming stem cells and mature white blood cells, called T cells, from the patients and genetically engineer them to carry a receptor for NY-ESO-1. When the modified stem cells and T cells are transfused back into the patients, they will target and kill tumors that carry the NY-ESO-1 protein on their surface.

"Few options exist for the treatment of patients whose cancers have metastasized due to resistance to current therapies," said UCLA Professor Antoni Ribas, M.D., Ph.D. "This clinical trial will allow us to try a new approach that engineers the body's immune system to fight metastasized tumors similar to how it fights germs and viruses."

This approach differs from a similar method, called adoptive T cell immunotherapy, which has already shown some limited promise in clinical cancer research. In adoptive T cell immunotherapy the mature T cells collected from patients in those studies eventually decline in number. As a result, the immune response against the cancer wanes over time and the cancer often returns. Because this clinical trial uses blood stem cells (which can make unlimited copies of themselves) in addition to the mature T cells, it provides patients with a continual supply of genetically modified T cells to fight off the cancer.

"Clinical trials using adoptive T cell immunotherapy have shown remarkable results in some patients," said Siwen Hu-Lieskovan, M.D., of the UCLA David Geffen School of Medicine and co-investigator in the trial. "Engineering a patient's own blood-forming stem cells to produce a continual supply of the T cells needed to attack cancer for a prolonged period could help us overcome the challenges and limitations with the current method."

"Backed by rigorous science, the stem cell derived T cell immunotherapy being developed by Dr. Ribas and his team has the potential to address advanced cancers that have spread beyond the primary tumor and which are associated with very low survival," says Maria Millan, M.D., CIRM's President and CEO (interim)."This trial is the first step in developing a therapy that could alleviate the complications resulting from cancer metastases as well as potentially improving outcomes in cancer patients where there are currently no effective treatment options. We are confident that CIRM's funding and partnership, in combination with the expertise provided by our Alpha Stem Cell Clinic network, will provide critical support for the successful conduct of this important clinical trial."

CIRM is currently funding a total of five clinical trials targeting cancer.

To learn more about this clinical trial, visit its page at clinicaltrials.gov. If you think you might be eligible to enroll, please contact Clinical Research Coordinator Justin Tran by email at justintran@mednet.ucla.edu or by phone at 310-206-2090.

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

Source URL: https://www.cirm.ca.gov/about-cirm/newsroom/press-releases/09112017/ucla-launches-cirm-funded-cancer-clinical-trial